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Grants Working Group Recommendations TRAN
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## **Mission Statement**



#### **OUR MISSION**

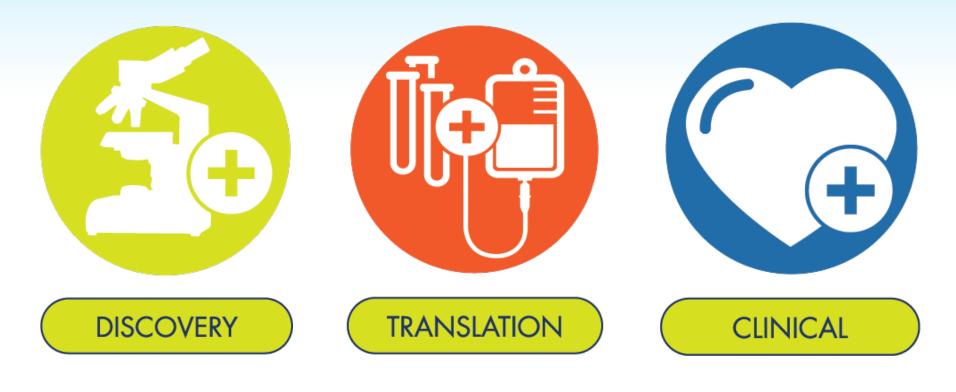
Accelerating world class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and world





# **Recurring Programs**





New Idea



Single Product Candidate



Pre-IND or Equivalent



Approved Therapy



# **Translation Program**



# TRAN1 Therapeutic

30 months \$4M (Cell/Biologic) \$2M (Drug) TRAN2
Diagnostic

24 months \$1.2M TRAN3 Medical Device

24 months \$2M TRAN4 Tool

24 months \$1M



# **Translation Program**



Disease modifying activity with candidate

## Proof of concept with prototype



TRAN1
Therapeutic

30 months \$4M (Cell/Biologic) \$2M (Drug)



TRAN2
Diagnostic

24 months \$1.2M



TRAN3
Medical
Device

24 months \$2M



TRAN4 Tool

24 months \$1M

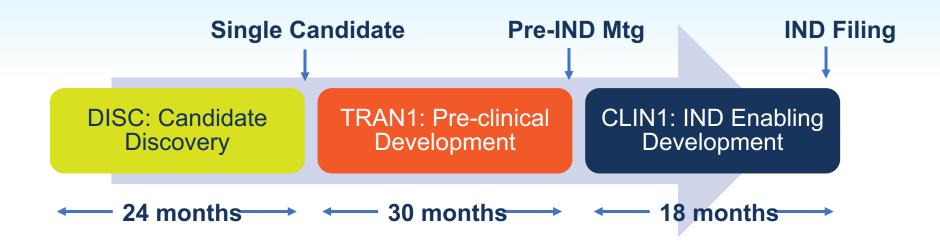


Design transfer to manufacturer

Completed Pre-IND or other Pre-Sub meeting with FDA







## Expected **outcomes** of TRAN1 applications:

- Pre-IND meeting conducted and formal meeting minutes forwarded to CIRM
- Pivotal IND-enabling preclinical studies protocols drafted
- GMP-compliant Master Cell Bank and Working Cell Bank established
- Clinical indication defined and draft clinical protocol synopsis developed



## **Review Criteria**



- Does the project hold the necessary significance and potential for impact? (i.e., what value does it offer; is it worth doing?)
- 2. Is the rationale sound? (i.e., does it make sense?)
- 3. Is the project well planned and designed?
- 4. Is the project feasible? (i.e., can they do it?)
- 5. Does the project address the needs of underserved communities?



## **Scoring System**



## Score of "85-100"

Recommended for funding, if funds are available

## Score of "1-84"

Not recommended for funding

Applications are scored by all scientific members of the GWG with no conflict.

The **median** of all individual GWG scores determines final score.





## **GWG** Composition and Roles



Scientific GWG Member



Scientific evaluation (disease area expert, regulatory, CMC, product development)

Provides scientific score on all applications

Patient Advocate or Nurse GWG Member



Patient perspective on significance and potential impact, oversight on process

Provides a DEI score

Scientific Specialist (non-voting)



Scientific evaluation (specialized expertise as needed)

Provides initial but not final scientific score



## **GWG** Recommendations



	Number of Apps	Total Applicant Request	Funds Available
Recommended for funding Score 85-100	4	\$16,232,581	\$70,400,000
Not recommended for funding Score 1-84	11		

For each award, the final award amount shall not exceed the amount approved by the ICOC Application Review Subcommittee and may be reduced contingent on CIRM's assessment of allowable costs and activities.







- Under Prop 14, any application that is not recommended for funding by the GWG, but which had 35% or more members score to fund the application must include a minority report.
- The minority report is included in the review summary and provides a brief synopsis of the opinion of reviewers that scored the application 85 or above.



# **CIRM Team Recommendations**



CIRM Team concurs with the GWG recommendations for funding.

In addition, the CIRM Team supports the minority position for application TRAN1-13996 and recommends funding of this application.

App Number	Title	Funds Requested	Score
TRAN1-13996	Overcoming resistance to standard CD19- targeted CAR T using a novel triple antigen targeted vector	\$4,168,679	83

